

FIG 1A

AAV-1	ttgccactccctctctgcgcgctcgctcgctcggtggggcctgcgggaccaaaggtccgc	60
AAV-2	...g.....ac..a....g.gc.....gc.	60
AAV-6	...g.....ac..a....g.gc.....gc.	60
Rep binding site		
AAV-1	agacggcagagctctgctctgcccggcccaccgagcgagcgagcgcgagagagggagtgc	120
AAV-2	c....c.c.g...t...c.g.g....t..gt.....	120
AAV-6	c....c.c.g...t...c.g.g....t..gt.....	120
TRS		
AAV-1	ggcaactccatcactaggggtaaTCGCGAAGCGCCTCCCACGCTGCCGCGTCAGCGCTGA	180
AAV-2	.c.....--..ct.G..G.-----TG.A...G----	163
AAV-6	.c.....--..ct.G..G.-----TG.A...G----	163
E box/USF		
AAV-1	CGTAAATTACGTCATAGGG---GAGTGGTCCCTGTATTAGCTGTCACGTGAGTGCTTTTGC	237
AAV-2	...G.....TTA.G.A.....AG.....-.....	222
AAV-6	...G.....TTA.G.A.....AG.....-.....	222
YY1 P5/TATA		
AAV-1	GACATTTTGCACACACCGTGGCCATTTAGGGTATATATGGCCGAGTGAGCGAGCAGGAT	297
AAV-2T...T..CGCT.....T..A.C.....AC.....G.	282
AAV-6T...T..CGCT.....T..A.C.....AC.....G.	282
YY1/p5 RNA Rep 78/68		
AAV-1	CTCCATTTTGAC-CGCGAAATTTGAACGAGCAGCAGCCATGCCGGGCTTCTACGAGATCG	356
AAV-2AG..G..GG.....C.....C.....G..T.....T.	342
AAV-6AG..G..GG.....C.....-.....G..T.....T.	341
AAV-1	TGATCAAGGTGCCGAGCGACCTGGACGAGCACCTGCCGGGCATTTCTGACTCGTTTGTGA	416
AAV-2T....C..C.....T...G...T....C.....AGC.....	402
AAV-6T....C..C.....T.....T....C.....AGC.....	401
AAV-1	GCTGGGTGGCCGAGAAGGAATGGGAGCTGCCCCGGATTCTGACATGGATCTGAATCTGA	476
AAV-2	A.....T...G..A.....	462
AAV-6	A.....T...G..A.....	461
AAV-1	TTGAGCAGGCACCCCTGACCGTGGCCGAGAAGCTGCAGCGCGACTTCCTGGTCCAATGGC	536
AAV-2T...ACGG.....	522
AAV-6G.....	521
AAV-1	GCCGCGTGAGTAAGGCCCGGAGGCCCTCTTCTTTGTTTCAGTTCGAGAAGGGCGAGTCCT	596
AAV-2T.....T.....G..A..T.....A..AG..	582
AAV-6	581
AAV-1	ACTTCCACCTCCATATTCTGGTGGAGACCACGGGGGTCAAATCCATGGTGCTGGGCCGCT	656
AAV-2A.G..CG.G..C....A....C....G.....TT....A..T.	642
AAV-6	641
AAV-1	TCCTGAGTCAGATTAGGGACAAGCTGGTGCAGACCATCTACCGCGGGATCGAGCCGACCC	716
AAV-2C.C..A..A..A.T....GA..T.....TT	702
AAV-6	701
AAV-1	TGCCCAACTGGTTCGCGGTGACCAAGACGCGTAATGGCGCCGGAGGGGGGAACAAGGTGG	776
AAV-2A.....C..A....CA.A.....C.....	762
AAV-6	761

FIG 1B

AAV-1	TGGACGAGTGCTACATCCCCAACTACCTCCTGCCCAAGACTCAGCCCCGAGCTGCAGTGGG	836
AAV-2T.....T...T.G..C....A..C.....T.....C.....	822
AAV-6	821
	P19/TATA	P19 RNA
AAV-1	CGTGGACTAACATGGAGGAGTATATAAGCGCCTGTTTGAACCTGGCCGAGCGCAAACGGC	896
AAV-2T.....AC.....T.....C.....G..T..CA.G.....T.....T	882
AAV-6C.....GG.....A.....G.....A..C..GG.C.....	881
AAV-1	TCGTGGCGCAGCACCTGACCCACGTCAGCCAGACCCAGGAGCAGAACAAGGAGAATCTGA	956
AAV-2	.G.....T.....G.....GTCG.....G.....A.....A..	942
AAV-6CG.....	941
	Rep 52/40	
AAV-1	ACCCAATTCTGACGCGCCTGTCATCCGGTCAAAAACCTCCGCGCGCTACATGGAGCTGG	1016
AAV-2	.T.....T.....G..G...A.A.....T..A..CA.G.....	1002
AAV-6A.....	1001
AAV-1	TCGGGTGGCTGGTGGACCGGGGCATCACCTCCGAGAAGCAGTGGATCCAGGAGGACCAGG	1076
AAV-2C.....AA...G..T.....G.....	1062
AAV-6	1061
AAV-1	CCTCGTACATCTCCTTCAACGCCGCTTCCAACCTCGCGGTCCCAGATCAAGGCCGCTCTGG	1136
AAV-2A.....T..G..C.....A.....T..CT...	1122
AAV-6	1121
AAV-1	ACAATGCCGGCAAGATCATGGCGCTGACCAAATCCGCGCCCGACTACCTGGTAGGCCCCG	1196
AAV-2G..A.....T..AGC.....T...A....C.....G....AGC	1182
AAV-6	1181
AAV-1	CTCCGCCCCGCGGACATTAAACCAACCGCATCTACCGCATCCTGGAGCTGAACGGCTACG	1256
AAV-2	AG..CGTG.A.....TCC.G...T..G..TAA..TT...A..A.....G....	1242
AAV-6C.....T.....	1241
AAV-1	AACCTGCCTACGCCGGCTCCGTCTTTCTCGGCTGGGCCCAGAAAAGGTTTCGGGAAGCGCA	1316
AAV-2	.T..CCAA..T..G..CT.....G..A.....AC.....A.....C...A.G.	1302
AAV-6	.C.....A..A....	1301
AAV-1	ACACCATCTGGCTGTTTGGGCCGGCCACCACGGGCAAGACCAACATCGCGGAAGCCATCG	1376
AAV-2T..A..T..C..G.....G.....A.	1362
AAV-6	1361
AAV-1	CCCACGCCGTGCCCTTCTACGGCTGCGTCAACTGGACCAATGAGAACTTTCCCTTCAATG	1436
AAV-2A.T.....G.....A.....C.	1422
AAV-6C.	1421
AAV-1	ATTGCGTCGACAAGATGGTGATCTGGTGGGAGGAGGGCAAGATGACGGCCAAGGTCGTGG	1496
AAV-2	.C..T.....G.....C.....	1482
AAV-6	1481
AAV-1	AGTCCGCCAAGGCCATTCTCGGCGGCAGCAAGGTGCGCGTGGACCAAAAGTGCAAGTCGT	1556
AAV-2G.....A.....A..A.....G..A.....C.	1542
AAV-6	1541
AAV-1	CCGCCCAGATCGACCCACCCCGTGATCGTCACCTCCAACACCAACATGTGCGCCGTGA	1616
AAV-2	.G.....A.....G..T.....	1602
AAV-6T.....	1601

FIG 1C

AAV-1	TTGACGGGAACAGCACCACCTTCGAGCACCAGCAGCCGTTGCAGGACCGGATGTTCAAAT	1676
AAV-2TCA..G.....A.....A.....	1662
AAV-6	1661
AAV-1	TTGAACTCACCCGCCGTCTGGAGCATGACTTTGGCAAGGTGACAAAGCAGGAAGTCAAAG	1740
AAV-2T.....G.....C..C.....	1722
AAV-6	1721
AAV-1	AGTTCTTCCGCTGGGCGCAGGATCACGTGACCGAGGTGGCGCATGAGTTCTACGTCAGAA	1796
AAV-2	.C..T.....G.....AA.....GTT.....A.....A.....A..	1782
AAV-6	1781
P40/TATA		
AAV-1	AGGGTGGAGCCAACAAAAGACCCGCCCGGATGACGCGGATAAAAGCGAGCCCAAGCGGG	1856
AAV-2G.....AG.....A...T...T.....A....	1842
AAV-6G.....	1841
P40 RNA		
AAV-1	CCTGCCCTCAGTCGCGGATCCATCGACGTCAGACGCGGAAGGAGCTCCGGTGGACTTTG	1916
AAV-2	TGC..GAG.....T...C.G.....---..T..A.CA...AC.	1899
AAV-6	1901
▼		
AAV-1	CCGACAGGTACCAAAACAAATGTTCTCGTCACGCGGGCATGCTTCAGATGCTGTTTCCCT	1976
AAV-2	.A.....T.....AA..T.....	1959
AAV-6	1961
AAV-1	GCAAGACATGCGAGAGAATGAATCAGAATTTCAACATTTGCTTCACGCACGGGACGAGAG	2036
AAV-2	...GACA.....CA..T..C.....T.....ACA..A..	2019
AAV-6	...A.....C....	2021
AAV-1	ACTGTTTCAGAGTGCTTCCCCGGCGTGTGAGAATCTCAACCGGTC---GTCAGAAAGAGGA	2093
AAV-2T.....T..---.....C..TTCT...GTC..A.A.G	2076
AAV-6A..T.....---.....	2078
AAV-1	CGTATCGGAAACTCTGTGCCATTTCATCATCTGCTGGGGCGGGCTCCCGAGATTGCTTGCT	2153
AAV-2A.....G..CTA.....A.CA....AAA..TG..A..--C.....A	2133
AAV-6	2138
Rep 78 stop		
AAV-1	CGGCCTGCGATCTGGTCAACGTGGACCTGGATGACTGTGTTTCTGAGCAATAAATGACTT	2213
AAV-2	.T.....T.....TT.....CA.C.T...A.....T..	2193
AAV-6T.....	2193
▽ VP1		
AAV-1	AAACCAGGTATGGCTGCCGATGGTTATCTTCAGATTGGCTCGAGGACAACCTCTCTGAG	2273
AAV-2	...T.....CT.....A	2253
AAV-6AC.....G	2258
AAV-1	GGCATTTCGCGAGTGGTGGGACTTGAAACCTGGAGCCCCGAAGCCCCAAAGCCAACCAGCAA	2333
AAV-2	..A..AA.AC.....A.GC.C.....CC.A..ACCA..A..GC..GCAG...GG	2313
AAV-6A.....	2318
AAV-1	AAGCAGGACGACGGCCGGGGTCTGGTGCTTCCTGGCTACAAGTACCTCGGACCCTTCAAC	2393
AAV-2	C.TA.....A..A.....T.....G.....	2373
AAV-6G..C.....G.....C.....	2378

FIG 1D

AAV-1	GGACTCGACAAGGGGGAGCCCGTCAACGCGGCGGACGCAGCGGCCCTCGAGCACGACAAG	2453
AAV-2A.....G.....A...A.....C.....A	2433
AAV-6T.....	2438
AAV-1	GCCTACGACCAGCAGCTCAAAGCGGGTGACAATCCGTACCTGCGGTATAACCACGCCGAC	2513
AAV-2G.....G.CAGC..A.....C.....CAA...C.....	2493
AAV-6A.AGCG..T.....T.....GCG...T.....	2498
AAV-1	GCCGAGTTTCAGGAGCGTCTGCAAGAAGATACGTCTTTTGGGGGCAACCTCGGGCGAGCA	2573
AAV-2	..G.....C..TA.....A.....	2553
AAV-6	..C.....T..GC.....G.....	2558
AAV-1	GTCTTCCAGGCCAAGAAGCGGGTTCTCGAACCTCTCGGTCTGGTTGAGGAAGGCGCTAAG	2633
AAV-2G..A...A.....T.....G..C.....CCT.T....	2613
AAV-6A.....T.T.....T.....	2618
<u>VP2</u>		
AAV-1	ACGGCTCCTGGAAAGAAACGTCCGGTAGAGCAGTCGCCACAAGAGCCAGACTCCTCCTCG	2693
AAV-2G.....A..GA.G.....C..T..TGTG.....	2673
AAV-6T.....G..AC.T.....G..G..ACAA.....	2678
AAV-1	GGCATCGGCAAGACAGGCCAGCAGCCCGCTAAAAAGAGACTCAATTTTGGTCAGACTGGC	2753
AAV-2	..A.C...A...G.G.....T..A.G...A...T.G.....A	2733
AAV-6T.....	2738
AAV-1	GA CTCAGAGTCAGTCCCCGATCCACAACCTCTCGGAGAACCTCCAGCAACCCCCGCTGCT	2813
AAV-2	...G....C.....A..T..C..C..G.....C.G..A.....G.....T...G.	2793
AAV-6	...T....G....C..C..C..A..A.....G.A..T.....A.....G.....	2798
<u>VP3</u>		
AAV-1	GTGGGACCTACTACAATGGCTTCAGGCGGTGGCGCACCAATGGCAGACAATAACGAAGGC	2873
AAV-2	C.....A...A...G.....A.....A.....G...	2853
AAV-6	2858
AAV-1	GCCGACGGAGTGGGTAATGCCTCAGGAAATTGGCATTGCGATTCCACATGGCTGGGCGAC	2933
AAV-2T...C.....A.....	2913
AAV-6	2918
AAV-1	AGAGTCATCACCACCAGCACCCGCGACCTGGGCCTTGCCACCTACAATAACCACCTCTAC	2993
AAV-2A.....C.....C.....	2973
AAV-6A..A.....T..C.....	2978
AAV-1	AAGCAAATCTCCAGTGCTTCAACGGGGGCCAGCAACGACAACCACTACTTCGGCTACAGC	3053
AAV-2	..A.....T....CCAA...---A...TCG.....T.....T.....	3030
AAV-6	3038
AAV-1	ACCCCTGGGGGTATTTTGATTTCACAGATTCCACTGCCACTTTTCACCACGTGACTGG	3113
AAV-2T.....C.....T..C.....	3090
AAV-6	3098
AAV-1	CAGCGACTCATCAACAACAATTGGGGATTCCGGCCCAAGAGACTCAACTTCAAACCTCTTC	3173
AAV-2	..AA.....C.....A.....G.....T	3150
AAV-6G.....	3158
AAV-1	AACATCCAAGTCAAGGAGGTCACGACGAATGATGGCGTCACAACCATCGCTAATAACCTT	3233
AAV-2T.....A.....CA.....C..TACG..G..G..T..C.....	3210
AAV-6G.....	3218

FIG 1E

AAV-1	ACCAGCACGGTTCAAGTCTTCTCGGACTCGGAGTACCAGCTTCCGTACGTCCTCGGCTCT	3293
AAV-2G..G..TA.T.....C.....G	3270
AAV-6T.G.....	3278
AAV-1	GCGCACCAGGGCTGCCTCCCTCCGTTCGGGCGGACGTGTTTCATGATTCCGCAATACGGC	3353
AAV-2T..A..A.....G.....A..A.....C.....G.G..A..G..T..A	3330
AAV-6G.....	3338
AAV-1	TACCTGACGCTCAACAATGGCAGCCAAGCCGTGGGACGTTTCATCCTTTTACTGCCTGGAA	3413
AAV-2C..C..G.....C..G..T..G..A..A.....C..T..A.....G	3390
AAV-6A.....G..A.....G.....	3398
AAV-1	TATTTCCCTTCTCAGATGCTGAGAACGGGCAACAACCTTTACCTTCAGCTACACCTTTGAG	3473
AAV-2	..C..T.....C.T..C..A.....T.....	3450
AAV-6A..G.....T.....C...	3458
AAV-1	GAAGTGCCTTTCCACAGCAGCTACGCGCACAGCCAGAGCCTGGACCGGCTGATGAATCCT	3533
AAV-2	..C..T.....T.....T.....T..C.....	3510
AAV-6	..C.....	3498
AAV-1	CTCATCGACCAATACCTGTATTACCTGAACAGAACTCAAATCAGTCCGGAAGTGCCCAA	3593
AAV-2G.....T...G.....AA.C.C..CAAGT....CCA..ACG	3570
AAV-6G.....G.....	3578
AAV-1	AACAAGGACTTGCTGTTTAGCCGTGGGTCTCCAGCTGGCATGTCTGTTTCAGCCCCAAAAC	3653
AAV-2	C.GTCAAGGC.T.A....TCT.AG.CCGGAG.GAG..A...TCGG.AC...T.T.GG...	3630
AAV-6G.....	3638
AAV-1	TGGCTACCTGGACCCTGTTATCGGCAGCAGCGGTTTCTAAAACAAAACAGACAACAAC	3713
AAV-2T.....C..C.....A..A.CA..G...TCTG.G..T.....	3690
AAV-6C.....	3698
AAV-1	AACAGCAATTTTACCTGGACTGGTGCTTCAAATATAACCTCAATGGGCGTGAATCCATC	3773
AAV-2TG.A.ACT.G.....A...A.C..G..CC.....CA.A..C..TC.G	3750
AAV-6C.....T.....T.....T..A	3758
AAV-1	ATCAACCCTGGCACTGCTATGGCCTCACACAAAGACGACGAAGACAAGTTCTTTCCCATG	3833
AAV-2	G.G..T..G..GC.C..C.....AAGC.....G.....T.....A.....T.....TCA.	3810
AAV-6A.....	3818
AAV-1	AGCGGTGTCATGATTTTTTGAAAAGAGAGCGCCGGAGCTTCAAACACTGCATTGGACAAT	3893
AAV-2G..TC.C..C.....G..GC.AG..T.A.AGAAAA....TGTGAACA.T..A..G	3870
AAV-6G.....	3878
AAV-1	GTCATGATTACAGACGAAGAGGAAATTAAAGCCACTAACCCTGTGGCCACCGAAAGATTT	3953
AAV-2CGG.A.A..C..T..C.....T..G..GCAG.A.	3930
AAV-6C.....C.....C.....	3938
AAV-1	GGGACCGTGGCAGTCAATTTCCAGAGCAGCAGCACAGACCCTGCGACCGGAGATGTGCAT	4013
AAV-2	..TT.T..AT.TAC...CC.....AG...A..G.C.AG.A..T....C.....CA.C	3990
AAV-6T.....C.....	3998
AAV-1	GCTATGGGAGCATTACCTGGCATGGTGTGGCAAGATAGAGACGTGTACCTGCAGGGTCCC	4073
AAV-2	A.ACAA..C.TTC.T..A.....C.....G..C.....T.....T.....G...	4050
AAV-6	T.....C.....A.....C.....A.....T	4058

FIG 1F

AAV-1	ATTTGGGCCAAAATTCCTCACACAGATGGACACTTTCACCCGTCTCCTCTTATGGGCGGC	4133
AAV-2	..C.....A..G.....A.....G..C.....T.....C.....C.....T..A	4110
AAV-6G.....C.....	4118
AAV-1	TTTGGACTCAAGAACCCGCCTCCTCAGATCCTCATCAAAAACACGCCTGTTCTGCGAAT	4193
AAV-2	..C.....T..AC....T.....A.....T.....G.....C..G..A.....	4170
AAV-6T...C.....	4178
AAV-1	CCTCCGGCGGAGTTTTTCAGCTACAAAGTTTGCTTCATTCATCACCCAATACTCCACAGGA	4253
AAV-2	...T..A.CACC..CAGT..GG.....C.....A..G.....G...	4230
AAV-6A.....G.....G..T.....	4238
AAV-1	CA-AGTGAGTGTGGAAATTGAATGGGAGCTGCAGAAAGAAAACAGCAAGCGCTGGAATCC	4312
AAV-2	..CG..C..C.....G..C..G.....G.....A.....	4290
AAV-6	..-.....C.....G.....A.....	4297
AAV-1	CGAAGTGCAGTACACATCCAATTATGCAAAATCTGCCAA-CGTTGATTTTACTGTGGACA	4371
AAV-2A.T.....T.....C..CAAC..G....TT..T...G..C.....C.....T.	4350
AAV-6T.....T...C.....-.....C.....	4356
AAV-1	ACAATGGACTTTTATACTGAGCCTCGCCCCATTGGCACCCGTTACCTTACCCGTCCTCTGT	4431
AAV-2	CT.....CG.G...T.A.....A.A.....G..T...AAT....	4410
AAV-6C.....	4416
	VP1-3 stop PolyA signal	
AAV-1	AATTACGTGTTAATCAATAAACCGGTTGATTCGTTTCAGTTGAACTTTGGTCTCCTGTCC	4491
AAV-2G.T.....T..A.....TGCGTA	4470
AAV-6GT.....A.....G.....A....G	4476
AAV-1	TTCTTATCTTATC-GGTTACCATGGTTAT-AGCTTACACATTA--ACTGCTTGTTGCGC	4547
AAV-2	..TC.T.....TA...T.....C..CGTAGA..AGT.GC.TGG.G.G..AA.CATTA	4530
AAV-6	..A.....T...C.....A.CA.C-C.G.....--.....A.....	4533
AAV-1	TTCGCGATAAAAGACTTACGTCATCGGGttacccttagtgatggagttgccactccctc	4607
AAV-2	ACTA.A.gg.a-----g.....	4570
AAV-6at.-----	4572
AAV-1	tctgcgcgctcgctcgctcggtggggccggcagagcagagctctgccgtctgcggaacctt	4667
AAV-2	.c.....ac..a.....gc..c..a..g..gc...a.gc.c.gg...	4630
AAV-6	.a.....g.....	4632
AAV-1	tggtccgcaggccccaccgagcgagcgagcgcgagagggagtgggcaa	4718
AAV-2	..cc.g.gc....t..gt.....c...	4681
AAV-6t.....	4683

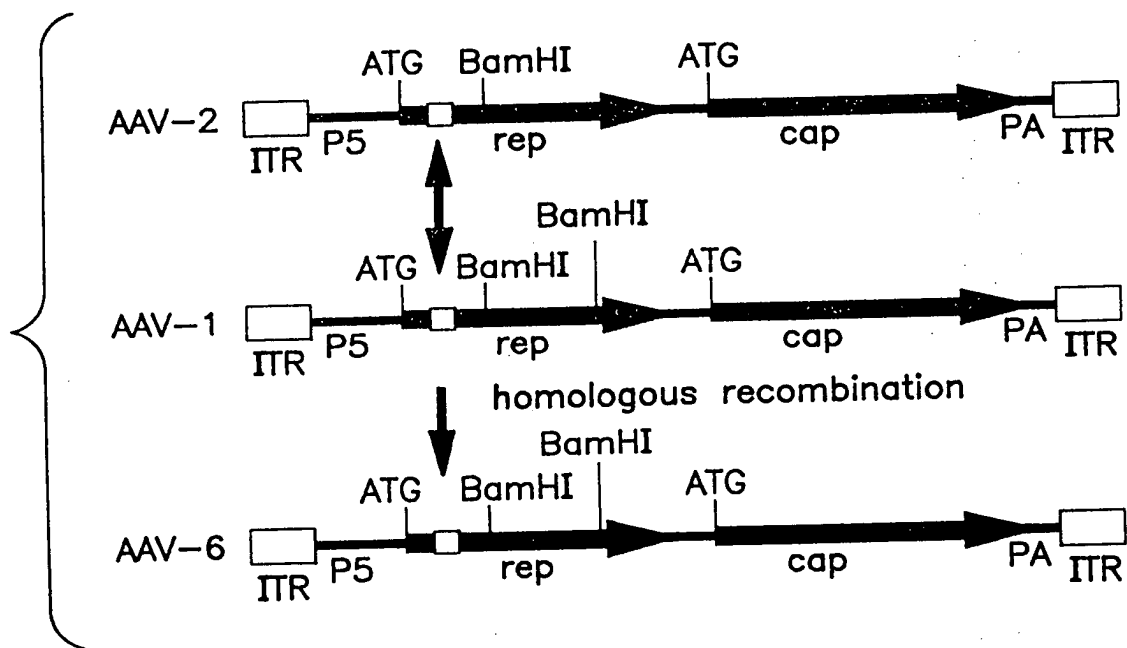


FIG. 3A

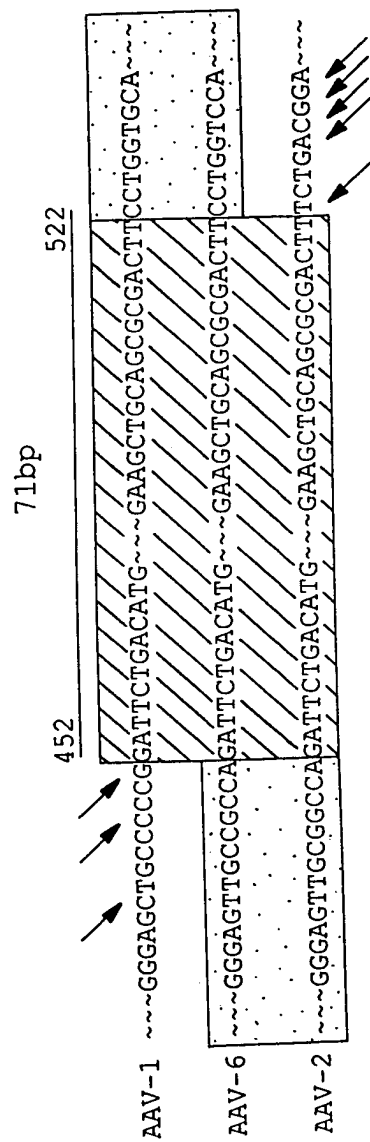


FIG. 3B

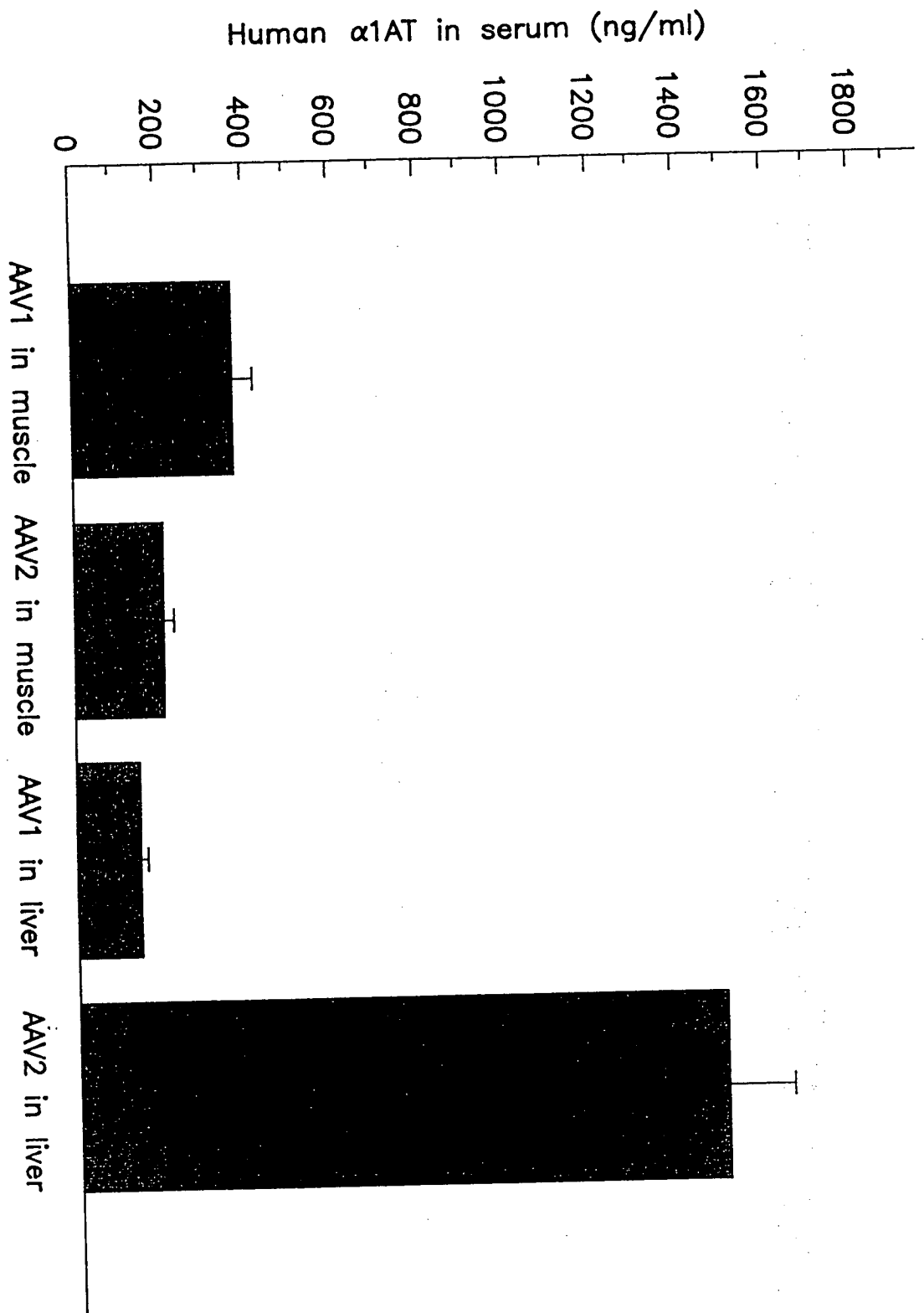


FIG. 4A

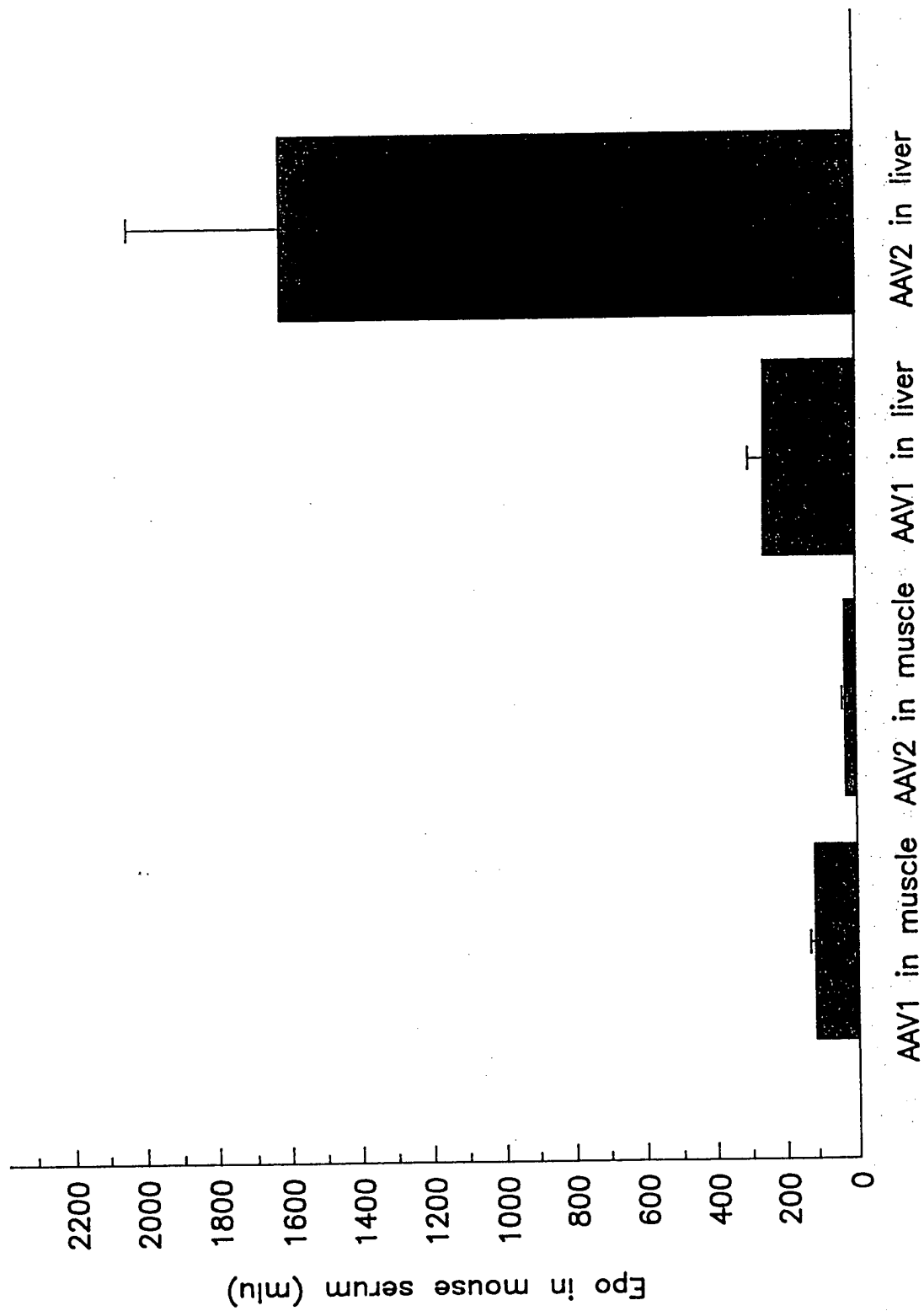


FIG. 4B

FIG. 5A

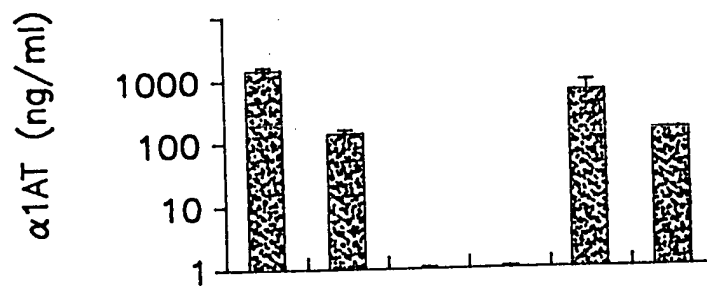


FIG. 5B

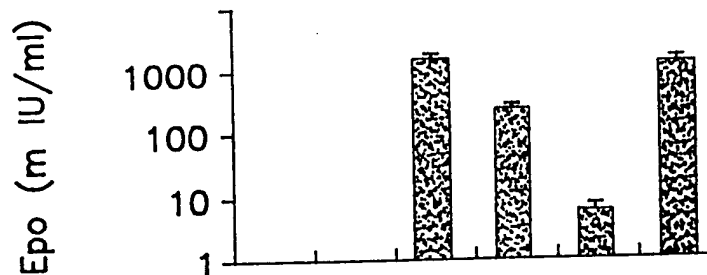


FIG. 5C

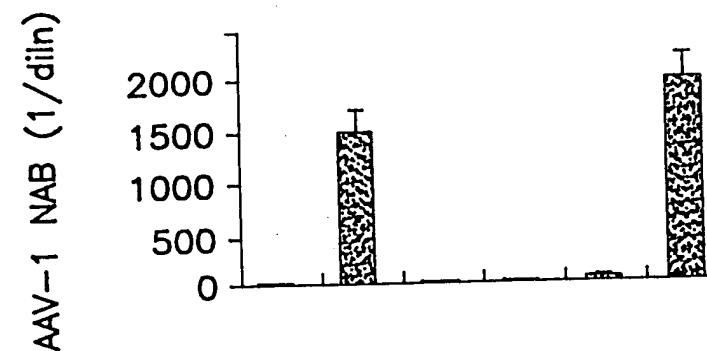
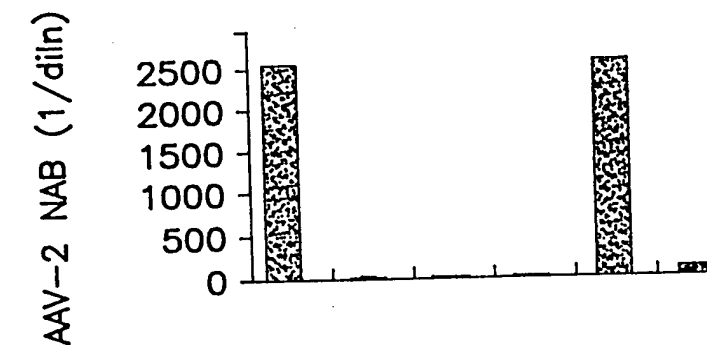


FIG. 5D



Group	1	2	3	4	5	6
Vector1-α1AT	AAV2	AAV1	PBS	PBS	AAV2	AAV1
Vector2-EPO	AAV2	AAV1	AAV2	AAV1	AAV1	AAV2

FIG. 6A

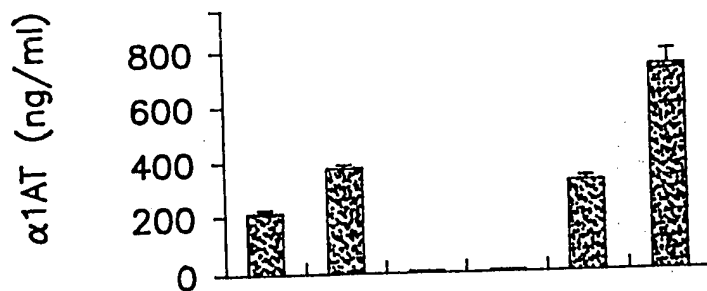


FIG. 6B

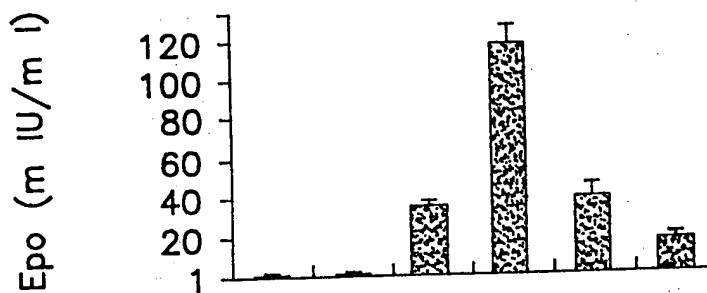


FIG. 6C

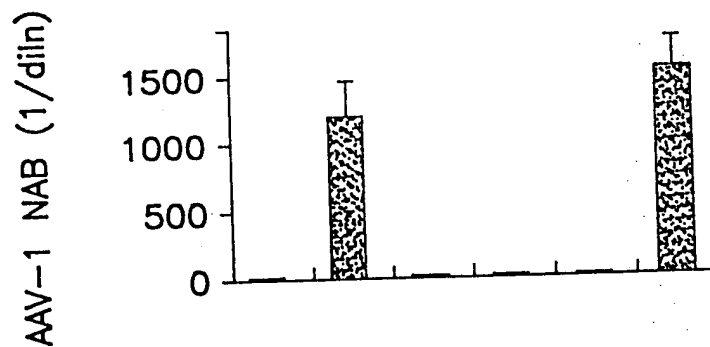
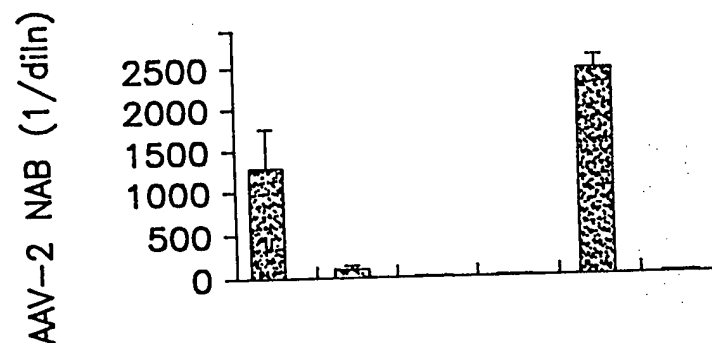


FIG. 6D



Group	1	2	3	4	5	6
Vector1 - α 1AT	AAV2	AAV1	PBS	PBS	AAV2	AAV1
Vector2 - EPO	AAV2	AAV1	AAV2	AAV1	AAV1	AAV2

A A A
A A A
G C
G C
T A
C G
gC Gc
cG Cg
C G
aA Tg a ca
G CCGGGTGGCTCGCTCGCTCGCGGCTCTCTCCCTCACCCGTT
a
---aav-1 itr
---aav-2 itr
---AAV-1 ITR
A C GGCCCCACCGAGCGAGCGCGCAGAGAGGGAGTGGGCAACTCCATCACTAGGGGTAA
G C t gt
cG Cg
C G
cA Tg
G C
gA Tc
G C
C G
T T
C
t

FIG 2